CIRM Board Approves Clinical Trials Targeting COVID-19 and Sickle Cell Disease

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The governing Board of the California Institute for Regenerative Medicine (CIRM) today approved new clinical trials for COVID-19 and sickle cell disease (SCD) and two earlier stage projects to develop therapies for COVID-19.

Dr. Michael Matthay, of the University of California at San Francisco, was awarded $750,000 for a clinical trial with mesenchymal stem cells for the treatment of Acute Respiratory Distress Syndrome (ARDS), a life-threatening manifestation of COVID-19 that leads to ICU admission and ventilator dependence. This award will allow Dr. Matthay to expand his current Phase 2 trial to additional underserved communities through the UC Davis site.

“Dr. Matthay indicated in his public comments that 12 patients with COVID-related ARDS have already been enrolled in San Francisco and this funding will allow him to enroll more patients suffering from COVID-associated severe lung injury,” says Dr. Maria T. Millan, CIRM’s President & CEO. “CIRM, in addition to the NIH and the Department of Defense, has supported Dr. Matthay’s work in ARDS and this additional funding will allow him to enroll more COVID-19 patients into this Phase 2 blinded randomized controlled trial and expand the trial to 120 patients.”

The Board also approved two early stage research projects targeting other manifestations of COVID-19.

- Dr. Stuart Lipton at Scripps Research Institute was awarded $150,000 todevelop a drug that is both anti-viral and protects the brain against coronavirus-related damage.

- Justin Ichida at the University of Southern California was also awarded $150,00 to determine if a drug called a kinase inhibitor can protect stem cells in the lungs, which are selectively infected and killed by the novel coronavirus.

The Board also awarded Dr. Pierre Caudrelier from ExcellThera $2 million to conduct a clinical trial to treat sickle cell disease patients

SCD is an inherited blood disorder caused by a single gene mutation that results in the production of “sickle” shaped red blood cells. It affects an estimated 100,000 people, mostly African American, in the US and can lead to multiple organ damage as well as reduced quality of life and life expectancy. Although blood stem cell transplantation can cure SCD fewer than 20% of patients have access to this option due to issues with donor matching and availability.

Dr. Caudrelier is using umbilical cord stem cells from healthy donors, which could help solve the issue of matching and availability. In order to generate enough blood stem cells for transplantation, Dr. Caudrelier will be using a small molecule to expand these blood stem cells. These cells would then be transplanted into twelve children and young adults with SCD and the treatment would be monitored for safety and to see if it is helping the patients.

“CIRM is committed to finding a cure for sickle cell disease, the most common inherited blood disorder in the U.S. that results in unpredictable pain crisis, end organ damage, shortened life expectancy and financial hardship for our often-underserved black community” says Dr. Millan. “That’s why we have committed tens of millions of dollars to fund scientifically sound, innovative approaches to treat sickle cell disease. We are pleased to be able to support this cell therapy program in addition to the gene therapy approaches we are supporting in partnership with the National Heart, Lung and Blood Institute of the NIH.”

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